

CLAIMS

We claim:

1. A method of treating motor neuron disease in a patient in need thereof, the method comprising delivering to a target site, a lentiviral vector pseudotyped with a rabies G envelope protein, the lentiviral vector comprising a nucleotide of interest (NOI), wherein the target site is at least part of the central nervous system, and wherein the NOI encodes a gene product that is expressed in the target site, thereby treating motor neuron disease in the patient.
2. The method of claim 1, wherein treating motor neuron disease comprises halting or delaying the degeneration of motor neurons in the patient.
3. The method of claim 1, wherein the delivery to the target site of the lentiviral vector comprising the NOI is by diffusion.
4. The method of claim 1, wherein the delivery to the target site of the lentiviral vector comprising the NOI is via intramuscular or intraparenchymal administration.
5. The method of claim 1, wherein the delivery to the target site of the lentiviral vector comprising the NOI is via retrograde transport.
6. The method of claim 1, wherein the motor neuron disease is ALS (Amyotrophic Lateral Sclerosis) or SMA (Spinal Muscular Atrophy).
7. The method of claim 1, wherein the target site comprises a target cell selected from the group consisting of a sensory neuron, a motor neuron, an astrocyte, an oligodendrocyte, a microglial cell, and an ependymal cell.
8. The method of claim 1, wherein the NOI encodes a neurotrophic or anti-apoptotic gene product.
9. The method of claim 1, wherein the NOI encodes a protein selected from the group consisting of SMN-1, GDNF, IGF-1, VEGF, XIAP, NIAP, and bcl-2.
10. The method of claim 1, wherein the lentiviral vector is pseudotyped with a mutant, variant, fragment or homologue of a rabies G envelope protein.
11. A method of delivering a nucleotide of interest (NOI) to a target site, comprising introducing a lentiviral vector comprising an NOI and pseudotyped with a rabies G envelope protein to the target site, wherein the target site is at least part of the central nervous system.

12. The method of claim 11, wherein the NOI can treat motor neuron disease by halting or delaying the degeneration of motor neurons in a subject.

13. The method of claim 11, wherein the NOI is introduced to the target site by diffusion.

5 14. The method of claim 11, wherein the NOI is introduced to the target site via intramuscular or intraparenchymal administration of the lentiviral vector.

15. The method of claim 11, wherein the NOI is introduced to the target site by retrograde transport.

10 16. The method of claim 12, wherein the motor neuron disease is ALS (Amyotrophic Lateral Sclerosis) or SMA (Spinal Muscular Atrophy).

17. The method of claim 11, wherein the target site comprises a target cell selected from the group consisting of a sensory neuron, a motor neuron, an astrocyte, an oligodendrocyte, a microglial cell, and an ependymal cell.

15 18. The method of claim 11, wherein the NOI encodes a neurotrophic or anti-apoptotic gene product.

19. The method of claim 11, wherein the NOI encodes a protein selected from the group consisting of SMN-1, GDNF, IGF-1, VEGF, XIAP, NIAP, bcl-2, and RAR β 2.

20 20. The method of claim 11, wherein the lentiviral vector is pseudotyped with a mutant, variant, fragment or homologue of a rabies G envelope protein.

21. A method of expressing a nucleotide of interest (NOI) in a target site, comprising introducing a lentiviral vector comprising an NOI and pseudotyped with a rabies G envelope protein to the target site, wherein the target site is at least part of the central nervous system, and wherein the NOI encodes a gene product that is expressed in the target site.

25 22. The method of claim 21, wherein expression of the gene product can treat motor neuron disease by halting or delaying the degeneration of motor neurons in a subject.

23. The method of claim 21, wherein the NOI is introduced to the target site by diffusion.

24. The method of claim 21, wherein the NOI is introduced to the target site via 30 intramuscular or intraparenchymal administration of the lentiviral vector.

25. The method of claim 21, wherein the NOI is introduced to the target site by retrograde transport.

26. The method of claim 22, wherein the motor neuron disease is ALS (Amyotrophic Lateral Sclerosis) or SMA (Spinal Muscular Atrophy).

5 27. The method of claim 21, wherein the target site comprises a target cell selected from the group consisting of a sensory neuron, a motor neuron, an astrocyte, an oligodendrocyte, a microglial cell, and an ependymal cell.

28. The method of claim 21, wherein the NOI encodes a neurotrophic or anti-apoptotic gene product.

10 29. The method of claim 21, wherein the NOI encodes a protein selected from the group consisting of SMN-1, GDNF, IGF-1, VEGF, XIAP, NIAP, bcl-2, and RAR β 2.

30. The method of claim 21, wherein the lentiviral vector is pseudotyped with a mutant, variant, fragment or homologue of a rabies G envelope protein.

31. The method of claim 21, wherein expression of the gene product treats or
15 prevents pain associated with a neurological disorder or injury.